

NF CONFERENCE

Major Announcement in Clinical Trials



Brigitte Widemann, MD

As hundreds of the world's leading NF experts convened in Monterey, California for the NF Conference, it was announced that for the first time in the history of the treatment of plexiform neurofibromas (PNs), over 50% of patients saw decreased volume in these inoperable tumors. In a presentation titled "The Promise of MEK: Therapeutics for NF1," Brigitte Widemann, MD, of the National Cancer Institute (NCI), reported that response data in a phase I trial for children and young adults is showing decreases in tumor volume. Anecdotal improvement in function and reduction in PN-related pain and disfigurement has also been observed.

Plexiform neurofibromas exhibit the most rapid growth in young children, and therefore early intervention in children with growing PNs may result in the greatest clinical benefit. Consistent volume decreases of large PNs have not been reported in the past, and this announcement indicates a promising future for the development of effective medical therapies for NF1-related PNs.

This highly successful clinical study is the result of a major investment of the Children's Tumor Foundation NF Preclinical Consortium (funded since 2008). Nancy Ratner, PhD and Wade Clapp, MD demonstrated that MEK inhibitors (MEKi) have a massive result on PN tumor volume in mice. The very robust effect of MEK inhibitors in mice with neurofibromas demonstrates the utility of the Ratner and Clapp NF animal models to select agents for clinical trials. A registration trial of selumetinib for inoperable PNs has been developed and will begin enrollment soon.

Continued on page 19

BIOMARKERS:

Children's Tumor Foundation and National Biomarker Development Alliance Join Together to Address Critical Research Need

The field of pediatric tumors suffers from a lack of biomarkers, and nowhere is this more evident than in children's tumors associated with neurofibromatosis (NF). The absence of standardized, measurable biological indicators (biomarkers) makes NF even more difficult to diagnose and treat, resulting in fear and frustration not only for patients, but also for the doctors and clinicians working to better their lives.

The Children's Tumor Foundation (CTF) and the **National Biomarker Development Alliance (NBDA)** have partnered to advance biomarker development in NF with potential for applications across other rare disease areas. The two organizations will work together to solve the problem of the absence of approved, measurable biological indicators for NF. Together they will focus on the discovery and development of biomarkers for pediatric brain tumors – with an emphasis on NF1-associated tumors.

What is a Biomarker?

Biomarkers are indicators in the body (blood, urine, normal or tumor tissue) that are "signals" that help scientists understand how the body functions normally and how these signals change in the presence of disease. These changes tell you to see your doctor. For example, if your blood pressure drops, your doctor may screen for heart disease. Some other types of biomarkers (such as changes in your body's molecules) can be used to diagnose cancer and other diseases. Biomarkers can also be used to monitor patients, as they can signal a tumor is coming back, even before it is obvious.

Nowhere are robust biomarkers more critical than in rare diseases such as NF. The data and processes developed through the NBDA and CTF collaboration will change the face of biomarker development, and will be publicly available to the broader community.

FOUR STARS

For the sixth year in a row, the Children's Tumor Foundation has been honored with a 4-star rating from Charity Navigator, America's largest independent evaluator of philanthropies. The Foundation is also accredited by the Better Business Bureau.



"Only 3% of the charities we rate have received at least 6 consecutive 4-star evaluations, indicating that the Children's Tumor Foundation outperforms most other charities in America," wrote John P. Dugan, Charity Navigator Founder and Chairman of the Board.

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nfregistry.org

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LETTER FROM THE PRESIDENT

Annette Bakker, PhD

At the Children's Tumor Foundation, we are all about strengthening our organization so that we can deliver results to those who are living with NF. They are our heroes; everything we do is for them.

Not long ago, we made a promise to dismantle the barriers that stand in the way of drug discovery. And now we are delivering on that promise: we are getting closer and closer to finding effective treatments.

Nowhere has this success been made clearer than at this year's NF Conference. Dr. Brigitte Widemann of the National Cancer Institute shared the results of a highly successful clinical study for NF1 in a presentation entitled "The Promise of MEK: Therapeutics for NF1."

Dr. Scott Plotkin shared results of clinical trials studying the effects of Bevacizumab on hearing loss for patients with NF2, which affected durable improvement in hearing for nearly 50% of involved patients.

The Foundation recently formed a partnership with the National Biomarker Development Alliance (NBDA). NBDA's co-director, former deputy-director of the National Cancer Institute, Dr. Anna Barker, is a world renowned specialist in biomarkers. Together with the NBDA, we will build a first-of-its kind center to develop biomarkers specifically for NF.

These are just a few of our recent accomplishments in the world of research; you can read more throughout this newsletter. But we are celebrating many other victories here at CTF: the success of the Volunteer Summit and Leadership Council, a powerful NF Forum, and a growing number of volunteers and donors.

We are also celebrating the success of our highly visible NF Awareness Month campaign, 'I Know A Fighter,' as well as our 'Shine a Light on NF' campaign, in which landmarks across the world lit up in blue and green to heighten awareness of NF.

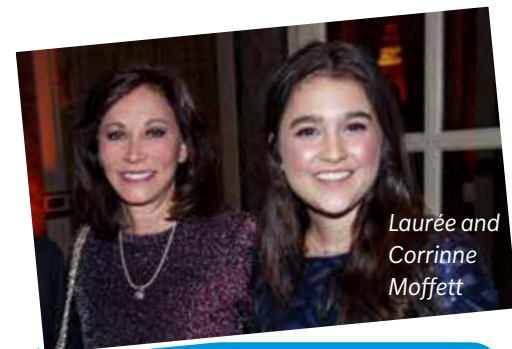
We enjoy our victories, but we push on! We have a goal: to end NF. Our vision is lofty, and as we grow, we will rely even more on your outstanding generosity. The matching gift funds for NF1, NF2, and schwannomatosis allow you to double any donation you make to the fight against NF at the Children's Tumor Foundation.

In order to win the fight against NF, we need you!

Gratefully,



Annette Bakker, PhD




Laurée and
Corrinne
Moffett

Thanks to the dedication and generosity of Jim Bob and Laurée Moffett, Carol Harrison, and Richard and Erica Horvitz, your donation towards all three types of NF can still be matched dollar-for-dollar. Please go to ctf.org/match to double your dollars for NF research.

Annual NF Conference

Over 300 of the top NF researchers and clinicians from more than 15 countries around the world gathered at the Portola Hotel and Spa in Monterey, CA from June 6th to 9th for the annual 2015 NF Conference. The four day event, which is the most important date on the NF research and clinical calendar, boasted a packed agenda that was both provocative and stimulating. Participants returned home with valuable new knowledge and relationships as a sure foundation for future, fruitful collaborations.




Anna Barker, PhD, Director of the National Biomarker Development Alliance (NBDA)


HIGHLY ACCLAIMED **KEYNOTE SPEAKERS IMPRESS** AT ANNUAL NF CONFERENCE

The Foundation's 2015 NF Conference kicked off with a special keynote speech delivered by **Anna Barker, PhD**, former deputy-director of the National Cancer Institute, and now professor at Arizona State University. Dr. Barker is also Director of the National Biomarker Development Alliance (NBDA), with whom the Foundation has recently forged an important collaboration to discover biomarkers specifically for NF. In her keynote address, "Biomarkers: The Best of Times and the Worst of Times," Dr. Barker gave her perspective on biomarker discovery and development, and outlined strategies that the NBDA and CTF will use to ensure efficient biomarker development for the NF community.

Professor of Neurology and Neuroscience and Director of the Brain Science Institute Drug Discovery Program **Barbara Slusher, PhD**, delivered the second keynote, "Academic-Industry Partnership in Drug Discovery for Neurological Diseases." Her talk delineated the changing landscape of drug discovery over the past decade: modifications in the drug research and development process have resulted in a significant rise in the number and sophistication of academic drug discovery centers. Dr. Slusher outlined details of the changing ecosystem and examples of academic-led drug discovery programs.



Barbara Slusher, PhD



David Bennett, MD, PhD

David Bennett, MD, PhD, Professor of neurology and neurobiology at the University of Oxford, delivered the third keynote speech. In his address, "Neuropathic Pain and Peripheral Nerve Repair," Dr. Bennett shared his current research on the pathogenesis of nerve injury in order to promote nerve repair and prevent the development of neuropathic pain. He presented a broad experimental approach to this problem, ranging from the study of transgenic mouse models to the investigation of patients with rare inherited neuropathies and painful channelopathies.

CLINICAL RESEARCH UPDATES: The Promise of BEV

There was an abundance of promising clinical findings presented at this year's NF Conference. Among them, **Scott Plotkin, MD, PhD** of Harvard Medical School/Massachusetts General Hospital presented "The Promise of BEV" about the use of Bevacizumab in treating vestibular schwannomas which cause hearing loss in NF2 patients.

A recent clinical study involving patients with NF2 revealed that Bevacizumab, an FDA approved drug that slows the growth of new blood vessels, has been effective in stabilizing the growth of vestibular schwannomas during treatment. To a lesser extent, Bevacizumab was also effective in affecting tumor regression. While Dr. Plotkin was quick to emphasize that Bevacizumab is not a miracle cure for vestibular schwannomas and other tumors, he underscored that it affected a durable improvement in hearing in nearly 50% of participants. Most impressively, during the yearlong study, not a single participant experienced additional hearing loss. A Phase-2 clinical trial of Bevacizumab for patients with NF2 vestibular schwannomas is enrolling now.

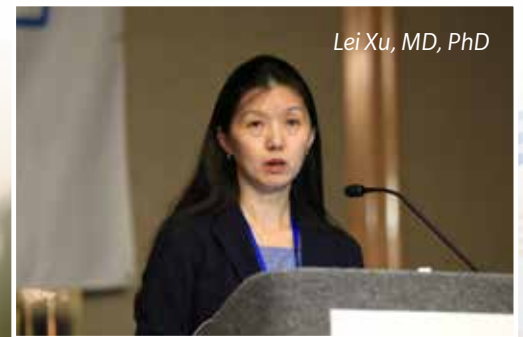
In a presentation given by **Lei Xu, MD, PhD** of Harvard University, additional data was presented on combination therapy of BEV and radiation to treat vestibular schwannomas.

For patients with sporadic vestibular schwannoma, radiation therapy is associated with high rates of local tumor control, but with significantly lower rates of hearing preservation. In contrast, treatment with Bevacizumab, as discussed by Dr. Plotkin, is associated with a reduction in the volume of growing vestibular schwannomas and with hearing improvement in some patients. As was demonstrated in two schwannoma xenograft models, the study showed that treatment exclusively with BEV creates a "normalization window" – a period during which tumor vessel vasculature is structurally and functionally close to normal – characterized by increased tumor perfusion and oxygenation.



Scott Plotkin, MD, PhD

The study found that radiation treatment is most effective when applied during this normalization window. This study is the first to demonstrate that combining BEV with radiation therapy is more effective than radiation alone in controlling schwannoma progression and improving neurologic function. This study was funded in part by a CTF Clinical Research Award (CRA) and a Drug Discovery Initiative Award (DDI).



Lei Xu, MD, PhD



NF CONFERENCE SATELLITE SESSIONS

In addition to the core agenda, morning pre-session satellite meetings were held to cover other important topics for specialized groups, including sessions specifically for NF Clinic Directors and Coordinators; a workshop on Autism Spectrum Disorder in NF; and a session for the REINS (Response Evaluation in Neurofibromatosis and Schwannomatosis) Initiative.

A special educational symposium was held for young clinicians the morning before the official start of the Conference. First introduced in 2013, it has become a very popular and worthwhile addition to the satellite meeting offerings. It has also become a tradition to hold optional “sunrise sessions,” where experts in the field of NF share their myriad experiences with young clinicians.

Collaborative groups also took advantage of their members’ attendance at the Conference. The CDMRP (Congressional Directed Medical Research Program), the International Schwannomatosis Database group, and members of Synodos for NF2 held meetings in the lovely Portola Hotel before and after the Conference.

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Autism Spectrum Disorder in NF1 – Satellite Workshop

Experts in neurofibromatosis and autism research gathered for a special satellite workshop to better understand the “social cognition” problems that people with NF1 face in their daily lives. Behavioral and cognitive issues are found in 50% of children with NF1 and can considerably impact the quality of life. As a consequence, children with NF1 are more vulnerable to social isolation, bullying, and loneliness and are less resilient than their peers. This is a common observation by parents and clinicians. However, in recent years, research has revealed that many of those children share behavioral traits also seen in children with Autism Spectrum Disorder (ASD).

Last year, researchers and experts in NF1 and autism combined efforts to develop a common agenda to better characterize this specific problem in NF1. **Maria Acosta, MD** from Children’s National Medical Center, an expert in cognitive and behavioral problems in patients with NF1, along with **John Constantino, MD**, world renowned expert in autism from Washington University in St. Louis, and **David Gutmann, MD, PhD**, one of the top experts in NF1, spearheaded a new effort to bring together researchers from both disciplines (NF and Autism) to develop a research agenda that can drive progress in this field.

The initial goal for this multidisciplinary, multicenter collaboration is to share clinical and biological samples as well as to combine the multiple areas of expertise to better characterize the social cognition deficits affecting children and their families with NF1.



Allan Belzberg, MD



Maria Acosta, MD

RESEARCH NEWS

REiNS International Working Group Meeting

The REiNS (Response Evaluation in Neurofibromatosis and Schwannomatosis) Initiative was established at the 2010 NF Conference with the goals of achieving consensus about the design and endpoints of future clinical trials for NF. REiNS is made up of seven working groups, focusing on imaging of tumor response; use of whole body MRI in NF; role of biomarkers in NF; and selection of functional outcomes, visual outcomes, patient reported outcomes, and neurocognitive outcomes. Initial recommendations were published in a supplement of the journal *Neurology*.

Several working groups have developed new recommendations which were presented at the meeting to receive critique and input from NF clinicians and researchers prior to submission for publication in a second supplement.



NF Clinic Directors and Coordinators Share Ideas

The NF Conference provided several opportunities for clinician interactions through the Children's Tumor Foundation NF Clinic Network (NFCN). Fifty-five clinic directors and coordinators shared their experiences, engaged in lively discussions, and worked together to develop strategies that will strengthen the clinic network.

Heather Radtke, MS, CGC, provided an update on NFCN efforts including the development of an NF symposium planning manual; NF clinic directory of NFCN members and specialists/referral database; communication/resource sharing strategies for clinicians; and program development ideas including recognizing and supporting the role of a clinic coordinator within an NF clinic.

Pamela Knight, MS, provided an overview of the Post-Mortem Biobank Program. The IRB-approved program works in conjunction with the National Disease Resource Interchange (NDRI), which provides tissue retrieval services for research. Next-of-kin or another designee will notify NDRI just before or after a patient dies. NDRI then retrieves NF-related tissues within 24 hours, sending them to a pathologist for quality control and assessment. The pathologist forwards the tissues to the CTF Biobank.

For additional information about this program, individuals may contact Pam Knight at pknight@ctf.org or 646-738-8555.



2015

Von Recklinghausen Award

Attendees managed to squeeze in a little bit of fun and leisure at the special Welcome Dinner and presentation of the prestigious von Recklinghausen Award. With the beautiful Pacific Ocean and stunning sunset as backdrop, the von Recklinghausen Award was presented to **Eric Legius, MD, PhD**, of the University of Leuven, Belgium. For over 20 years, Dr. Legius has dedicated himself to the understanding of NF1 and to the advancement of patient care. Considered by many as the most influential NF1 clinician/scientist in Europe, Dr. Legius also lends a strong spirit of collaboration to the NF clinical and research community, organizing stimulating NF workshops, and sharing his broad insights, from genetics, cancer, pediatrics, psychology, and basic cell biology.



2015 Young Investigator Award Recipients

LEI XING is a fifth-year postdoctoral fellow at the University of North Carolina, School of Medicine.

MAPK/ERK Hyperactivation on Neural Circuit Development in NF1

Dr. Xing will test the effect of layer V neuron activation in a genetic mouse model with upregulated ERK/MAPK signaling, which should mimic the effect of reduced neurofibromin activity. He hypothesizes that this may help us understand, and potentially be a marker for, the presence of NF1 features such as cognitive and psychomotor delays.



The Children's Tumor Foundation is pleased to announce the funding of six Young Investigator Awards (YIA) for 2015. The YIA is the Foundation's oldest research award program and serves to advance understanding of the biology of NF1, NF2, and schwannomatosis. Of the six awardees, three are pre-doctoral students and three are post-doctoral fellows. The title of the awardees' application indicates the focus of the research that will be funded through this award.

MARISA ANN FUSE is a predoctoral student at the University of Central Florida.

In Vivo Testing of FDA-Approved Drugs for NF2

The project leverages the work already being done in screening drug libraries, focusing on the potential PI3K and mTOR inhibitors that kill/suppress NF2-deficient schwannoma cells in vitro. This student will then test these drugs for effectiveness.



EBRAHIM TAAHEI SEYEDMOHAMMAD is a predoctoral student at Vanderbilt University.

The Inhibitory Role of Integrin beta3 in NF1 Impaired Osteogenesis

This student will use a tibial dysplasia mouse model to test whether the increased integrin beta3 that he found in NF1-deficient osteoprogenitor cells prevents them from differentiating into osteoblasts, leading to the failure of NF-related bone lesions to heal. He will subsequently test whether knocking down integrin beta3 production can lead to increased fracture healing.



VANESSA MERKER is a predoctoral student at Massachusetts General Hospital.

Coordinating Care for Patients with Schwannomatosis - Assessing the Field and Identifying Opportunities for Improvement

This student will investigate how schwannomatosis patients navigate the health care system with the long term goal of educating the medical community and patient population to improve patient access to appropriate medical care.



DIPAK N. PATIL is a third-year postdoctoral fellow at the Scripps Research Institute.

Understanding the G Protein Coupled Receptor (GPCR) Driven Interaction of NF1 with G Proteins

This project will perform experiments based on observations that activated GPCRs can inhibit neurofibromin's GAP activity by binding G protein subunits, which will lead to better understanding of the molecular mechanism involved in this important signaling regulation. This could lead to new targets for NF1 therapies.



AUBIN MOUTAL is a second-year postdoctoral fellow at the University of Arizona (Tucson).

Molecular Targeting of Migraine in the NF1 Population.

Dr. Moutal will study the interaction of the CRMP2 protein and neurofibromin, to determine whether its perturbation (by reduction of neurofibromin) could be related to migraine in patients with NF1. This work could lead to therapies specifically effective in NF1 or be of general use for migraine headache (or potentially be useful in understanding other NF1 features).



NF ENDURANCE



Summer Solstice

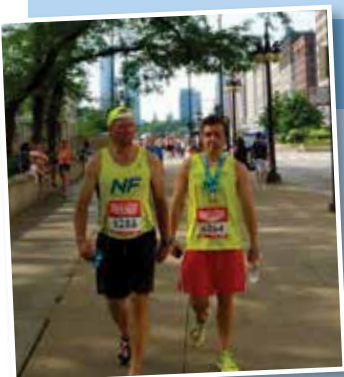
The 3rd annual Summer Solstice 10k and Kids Fun Run took place in June in Spokane, Washington. Event chair Janet Carney brought together over 150 participants for the race! A big thanks to event sponsor Fleet Feet Sports Spokane. In the past three years, this event has raised over \$35,000. If you are interested in hosting a 10k, 5k, or Kids Fun Run in your community, please let us know at nfendurance@ctf.org.

CHOOSE YOUR OWN EVENT

New York

Diane & Jeff Owens

Two years ago Diane & Jeff Owens' four month-old son, Alex, was diagnosed with NF1. Told by doctors that it was simply a "wait-and-see" situation, Diane knew "doing nothing was NOT an option!" This year, Diane ran the Long Island Marathon and Jeff planned his first benefit golf outing. Together they have raised almost \$61,000, and they are not done! This November, Diane will be running in the NYC Marathon.



Chicago Eddie Purtell

Since 2012, Eddie Purtell of Milwaukee, Wisconsin has raised over \$18,000 by running the Rock 'n' Roll Chicago Half Marathon. Eddie is a full-time college student but he still has time to train! This year, Eddie is on pace to break through his 2014 goal and raise over \$11,000.

IRONMAN KONA

Thanks to our partnership with IRONMAN, Michael Rasco, Bryan Putnam, and Elizabeth Dewberry will be representing the NF Endurance team in the exclusive IRONMAN World Championship event in Kona, Hawaii. Each athlete has committed to raising at least \$40,000 for the Foundation.



Elizabeth Dewberry, (top); Michael Rasco, (right) and Bryan Putnam (bottom)

UPCOMING EVENTS

9/19 **Little Heroes 5k | Pennsauken, NJ**

9/27 **Berlin Marathon | Berlin, Germany**

10/10 **IRONMAN Kona | Kona, HI**

10/11 **Bank of America Chicago Marathon | Chicago, IL**

11/1 **TCS New York City Marathon | New York, NY**

11/7 **IRONMAN Florida | Panama City Beach, FL**

12/6 **California International Marathon | Sacramento, CA**

Visit nfendurance.org for more information.

RACING 4 RESEARCH

Thirty NF families from across North America joined CTF at the racetrack during the month of May as a part of our Racing4Research program. May NF Awareness Month started off with a bang in Northern California for Park Place Motorsports, with the team capturing their first pole position and first win in the No. 73 Children's Tumor Foundation/Justice Brothers Porsche. Drivers Patrick Lindsey and Spencer Pumpelly both wore suits featuring the CTF logo.

The weekend was also the debut of Dr. Jim Norman's new Lamborghini Huracán, which sports the CTF logo. The logo clearly brought Dr. Jim and his 20-year-old son Josh good luck: they won their first-ever race together in the Super Trofeo series. It turns out the Normans are just as compassionate as they are competitive: Jim's wife, Gail, a nationally ranked triathlete, will be joining our NF Endurance team for IRONMAN Kona, competing and fundraising on behalf of CTF.

On the other side of the country, NF Heroes were also atop the podium, this time with Compass360 Racing at the Canadian Tire Motorsport Park. The C360 squad brought home an astounding three trophies in one weekend, all in their two Children's Tumor



Foundation Art Cars. Local NF Heroes joined drivers Paul Holton and Emilee Tominovich in Victory Lane.

All NF families are welcome to join Racing4Research at the racetrack—enjoy a fun day at the track, compliments of our race partners. To learn more, go to racing4research.org to see a schedule of upcoming events, and contact Jill Beck at jbeck@ctf.org to arrange a visit at a track near you!

RACE ACROSS AMERICA



Congratulations to the four women who participated in the Race Across America (RAAM) event on behalf of the Children's Tumor Foundation. Team members included CTF board member Lesley Oslica, Julie Hathcock, Hannah Turnbough, and NF Endurance staff member Angela Earle. These athletes rode constantly for 8 days and 18 hours, covering over 3,000 miles and crossing 12 states. From high temps to high hills, encountering big blisters and bold bears, the NFE team crossed the finish line in Annapolis, Maryland after starting in Oceanside, California.

"In addition to the awareness gained, our team has raised over \$76,000 so far – which will be matched by the Moffett Family Match Fund to become more than \$150,000 for NF Research. But there is one goal that has not been met... and that's to end NF. We will keep fighting, riding, running, walking, and fundraising," said Lesley Oslica.

Thank you, team, for raising awareness and funds for CTF across the United States.

NF WALK



Staten Island



San Jose



Providence



Cincinnati



Boston



Kernersville



Scottsdale



Philadelphia

THANKS TO OUR WALK COMMITTEES

One of the best ways to grow an NF Walk from a small, intimate event with family and friends, to a huge celebration for hundreds of people is to get new volunteers to join the planning committee. By adding a nearly 20-person committee to this year's Iowa NF Walk, we spread awareness to a new audience, increased donations, and pulled in more participants than ever before. Thanks to the growth of the Cincinnati NF Walk committee this year, the event was able to go from \$30,000 last year to nearly \$50,000 in 2015!

NF WALK EVERYWHERE

Starting this September, you can **Walk Everywhere!** Don't see an official NF Walk in your city? Then join us virtually! You pick the day and the place and we'll give you the tools to register, invite your friends, and fundraise. Do you already walk with friends? Could your office use a teambuilding lunch hour? Then why not pick a day to walk to raise awareness and funds for NF research? To learn more about this new ongoing national event, please visit our website at nfwalk.org.



JOIN US AT AN NF WALK!

Meridian, ID	September 12
Kansas City, MO	September 12
Biddeford, ME	September 12
Seattle, WA	September 12
Moorhead, MN	September 13
New York, NY	September 19
Jacksonville, FL	September 19
Addison Oaks, MI	September 20
Denver, CO	September 26
Putnam, CT	September 26
Corbin, KY	September 26
Gastonia, NC	September 26
Central NJ	September 26
Columbia, SC	October 3
Dallas, TX	October 3
Richmond, VA	October 3
Rochester, NY	October 3
Columbus, OH	October 4
Orlando, FL	October 10
Phoenix, AZ	October 11
Atlanta, GA	October 17
Hoover, AL	October 18
Long Island, NY	October 18
Las Vegas, NV	October 24
Los Angeles, CA	October 25
Nashville, TN	October 25
Washington, DC	October 25
Houston, TX	November 7
Boca Raton, FL	November 7

FOUNDATION NEWS

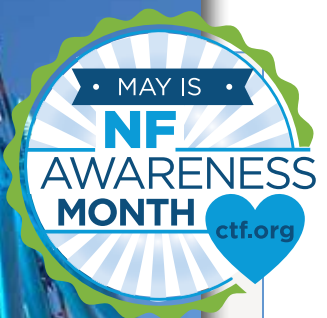
Awareness Month HIGHLIGHTS

May is NF Awareness month and this year the Children's Tumor Foundation launched a new campaign, "I KNOW A FIGHTER," to draw attention to the inspirational and remarkable stories of those living with NF, who are fighters in every sense of the word.

Members of the NF community were instrumental in expanding the reach of I Know a Fighter, bringing NF awareness into their communities through the **Shine a Light on NF** campaign; working with their local government on a city or state proclamation; organizing fundraisers; spreading awareness about NF through the media; submitting videos; and sharing content online.

We thank all of our partners and friends throughout the world who promoted NF Awareness Month, with additional recognition to the following:

- Bedrock Management
- Coastal Outdoor Advertising
- Cleveland Cavaliers
- Dan Gilbert
- Danica McKellar
- Made Strong



Seattle Great Wheel, Seattle WA



The Z, Detroit MI



Wells Fargo Duke Energy Center, Charlotte NC



Millburn, NJ

MEET THE STAFF

Kristine Poirier



HOMETOWN:
Weymouth, MA

CURRENT TOWN:
Norwell, MA

EDUCATION:
Bachelors in fashion design from Syracuse University.

WORK WITH THE FOUNDATION:
As Program Director for Community Relations, I lead a team of five Community Relations representatives throughout the United States. Community Relations is responsible for supporting our local volunteers, developing community events, and cultivating relationships.

FAVORITE EXPERIENCE WITH THE FOUNDATION:
My daughter has NF1 and I have been involved with the Foundation for 15 years. My favorite experience with the Foundation is definitely the New England Gala, where I started with the Foundation as a volunteer. I have been a part of the New England Gala committee since I started and the committee members are my NF family.

FAVORITE HOBBY: Sewing--I made my sister's wedding gown--and anything outdoors: running, biking, hiking.

FAVORITE MOTTO OR APHORISM: "Actions Speak Louder Than Words."

FAVORITE BREAKFAST FOOD: French toast & bacon

IF YOU COULD HAVE ANY SUPERPOWER, WHAT WOULD IT BE? Invisibility

GO TO KARAOKE SONG: Never done it! Only something for me and the shower walls.

MEET THE BOARD

Connie Sorman



HOMETOWN: I was born in New Haven, CT and moved to Rochester, NY after graduating from college in 1987.

CURRENT TOWN: Richmond, Virginia

EDUCATION: I attended Marist College in Poughkeepsie, NY for my undergraduate degree in fashion design and merchandising, with a concentration in French.

WORK WITH THE FOUNDATION: I am currently the Volunteer Leadership Council chair. I am leading a group of about 150 active volunteers, fundraisers, and advocates across the country. Previously, I served as the Chapter Council vice chair and the Western New York and Virginia Chapter leader, respectively. I have also chaired three NF Walks, helped on a Registry Task Force, advocated on Capitol Hill, participated in Racing4Research, volunteered with Cupid's Undie Run, contributed on the 2015 NF Forum planning committee, and served on an NF Walk committee in Richmond.

FAVORITE EXPERIENCE WITH THE FOUNDATION: My work with the Foundation volunteers is what gives me the most fulfillment. I feel that I have a greater impact when I get to guide and organize volunteers who are actively working and supporting those within their own communities.

FAVORITE HOBBY: I love to travel and to spend time with family. I enjoy creative writing and write a blog for NF awareness called NFSaid.blogspot.com.

FAVORITE MOTTO OR APHORISM: "Life is like a box of chocolates; you never know what you're gonna get."

FAVORITE BREAKFAST FOOD: Vegan smoothie bowls with homemade granola

IF YOU COULD HAVE ANY SUPERPOWER, WHAT WOULD IT BE? My super power would be to destroy worry and fear!

NEW FACES AT THE CHILDREN'S TUMOR FOUNDATION

Welcome to the Foundation's newest staff members!



Paul Dranginis
Director of Major Gifts
pdranginis@ctf.org



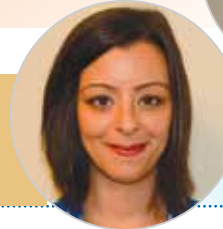
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rdeornelas@ctf.org

MEET AN NF HERO: *Loric Colgrove*

"Hi there! My name is Loric and I was born with NF1. I am 10 years old and until recently NF has only affected my life by giving me café au lait spots. Then, an MRI of my spine and brain showed a marble-sized tumor near my cerebellum. The doctors say that I am lucky because the tumor was in an accessible place and they were able to remove it surgically.

"NF is just a part of me. I am a normal boy who loves to ride bikes, rollerblade, tumble, dance, swim, and wrestle with my siblings. I also have a nickname, 'Lego Boy Loric,' because I LOVE LEGOS!! Especially vehicles! I will stay up late at night just to build them.



"It is my hope that I can help people learn about neurofibromatosis through my experiences, and make some new friends as well!"

What is your idea of the perfect day?

Riding my bike and building LEGOs all day.

What is your favorite breakfast food?

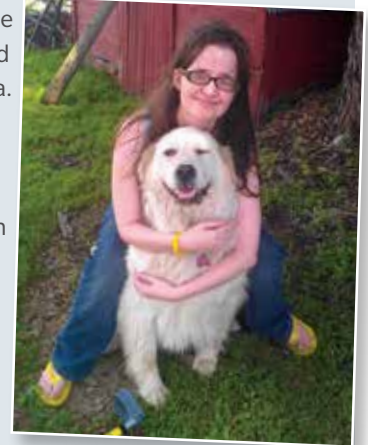
Sausage patties (That's all I would eat during my hospital stay after surgery.)

What sound or noise do you love? When our chickens find food and start clucking for the others to come and share it with them.

If you could have any superpower, what would it be? I would love to be able to fly so that I could catch birds in the air and rescue cats from trees.

STORIES OF NF: *Nora Wade*

"My name is Angelique but I prefer to be called by my nickname, Nora. I was diagnosed with NF at Nationwide Children's Hospital in Columbus, Ohio when I was three years old, and am now seen at the University of Minnesota Medical Center.



"I am 27 years old. I have several doctor appointments each month, I have had many surgeries, I have rods in my back, and I live with constant pain. But it doesn't change the fact that I live and I love. It doesn't change the fact that I'm a fighter. It doesn't change the fact that I still love a good cup of coffee and laughing with friends.

"People often tell me that they're sorry that I have NF. I'm not sorry. Having NF has taught me so much. It's taught me how precious life is and that it can change at any given moment. It's taught me how to be a fighter, to be my own hero, to be a champion. It's taught me that it's okay to take each day as it comes for I never know what tomorrow holds.

"NF may currently have no cure, but that doesn't mean there is no hope. I fully believe that I can still achieve my life goals and dreams, albeit on a different timeline than what I had originally envisioned. I may have NF, but NF doesn't have me!"



NORD Award

Congratulations to 2014 CTF Ambassador **Bailey Gribben**, who was recently honored by the National Organization of Rare Diseases (NORD) at their Portraits of Courage Gala. Bailey was one of ten patients or caregivers who were honored for making a positive impact on the lives of others and for the rare disease community. Standing next to Bailey in the above photo is the Director of the National Institutes of Health (NIH) and a co-discoverer of the NF gene, Dr. Francis Collins.

IN THE MEDIA

Annette Bakker, PhD, President and Chief Scientific Officer at the Children's Tumor Foundation, wrote "Uprooting the Foundation," an article about innovation, collaboration, and fighting back against rare diseases for the April issue of *NonProfit PRO*. To read the article, please go to ctf.org/bakker.



COMMUNITY NEWS

Great Events from Across the NF Community

The Foundation has a presence across the United States and facilitates local patient support groups, medical symposia, and fundraising events. Learn more about the Children's Tumor Foundation in your area by visiting www.ctf.org/communityrelations.

GEORGIA

On June 15th, the 4th annual CureNFwithJack golf tournament was held at The Manor Golf Course in Alpharetta, GA. Over 150 people attended the event!



CONNECTICUT

John Lipof organized a Spinathon at Edge Fitness in Fairfield, CT. The results were spintastic! Fifteen attendees raised over \$11,000 for NF2 research. Thanks to Edge Fitness for donating the spinning room!



RHODE ISLAND

The Providence NF Walk, which took place on May 31st, was a day filled with fun, food, and lots of walking. With over 75 participants, the event raised a total of \$17,000 for CTF. Event activities included face painting, a Kids Fun Run, a DJ, and food from Dunkin' Donuts.

NEW YORK

The NYC Poker Tournament was held on June 11th and raised over \$100,000. Organized by a committee led by Dan Altman and supported by Marisa Casper, John Tolpin, Eric Altman, San Betzag, Ron Palmese, Garrett Gleeson, and Ken Goodkind, the tournament ended with a heartwarming surprise. The grand prize was an entry into the World Series of Poker (WSOP), which was won by Eun Yuster. Both Yuster and runner-up Matt Gervasio decided to pass this coveted entry on to the third place winner, Ken Goodkind.

Ken Goodkind is an NF2 dad and a longtime CTF contributor whose son Adam was scheduled to undergo surgery near the upcoming WSOP event, making this family's trip out West a much happier experience for all.



From left: Julie Pantoliano (Children's Tumor Foundation), Ken Goodkind (3rd place winner), Matt Gervasio (2nd place winner), Eun Yuster (1st place winner)



ALASKA

The 35th annual Tok Trot was held in Tok, AK on April 18th, and raised over \$500 through donations and T-shirt sales. Some of the bright blue CTF shirts had to be worn under coats due to the cold Alaskan wind! Local NF Hero Holly was even honored with a blue ribbon.



CALIFORNIA

NF Hero Blake Robinson's aunt, Kathy Folk, and her friend, Dolores Trowbridge, hosted a wine and art event in honor of Team Blake. Local artists were invited to show and sell their artwork, with a percentage of all sales donated to CTF. The event raised \$3,000 for the Los Angeles NF Walk.

FLORIDA

Bluewater Movements will host their annual Saltwater Fishing Tournament series, which includes three tournaments in Pompano Beach, FL. A portion of the money from these tournaments benefits CTF.



COLORADO

"Iron Butt" Rides Again - 1,000 miles! That's how far the "Iron Six" motorcyclists rode straight through from Denver, CO to DeKalb, IL on June 30th for the annual "Iron Butt" motorcycle ride to benefit CTF.

GEORGIA

On June 30th, CTF participated in NF Night at Turner Field. Washington Nationals shortstop Ian Desmond hosted NF families at the ballpark. Over 30 people accepted Desmond's invite, spreading NF awareness while taking in both batting practice and the game! Thanks Ian!



COMMUNITY NEWS



UTAH

The 2nd Annual #EndNF with Travis Classic Golf Tournament took place at the Ridge Golf Club in Salt Lake City. One hundred golfers joined the event, which included an awards banquet, raffle, and silent auction. In-kind donations totaling over \$37,000 were distributed throughout the event, allowing everyone to walk away a winner.



Travis Carpenter shares a laugh with his Orthopedic Surgeon, Dr. Steven Santora, who operated on Travis two days after this event at Shriners Hospital for Children in Salt Lake City.

CONNECTICUT

More than 60 sixth graders at Bennet Academy in Manchester, Connecticut worked alongside "Team Inspired by Ryan" to fundraise and increase awareness of NF in what has become an annual event. The students organized a raffle consisting of nine goody-filled baskets, including items from Carla's Pasta of South Windsor and Highland Park Market.



MASSACHUSETTS

Local NF Hero Ben Aliber and his friend Greg Ward collaborated to produce a musical at Christ Episcopal Church to fund raise for CTF. The musical, written by 8th grader Ward, is called "Together Forever." Aliber starred in the piece, which featured themes of acceptance, support, and love. Their production raised \$1,960 for NF research!



FLORIDA

The first annual "A Night in Monte Carlo" event took place at the Omni Amelia Island Plantation Resort. Over 80 guests enjoyed an evening of poker, blackjack, and other entertainment, raising over \$15,000 to support CTF.



NEVADA

The University of Nevada, Las Vegas Student Union & Event Services Department adopted CTF this year, raising funds through a variety of events including a pancake breakfast and a "Pie Your Boss" event. At a community gathering on May 19th, local NF Hero Elyana Bjorkquist accepted a check on behalf of CTF. UNLV is also the host site of the Las Vegas NF Walk on October 24th.

BACK TO SCHOOL

KIDS PROGRAMS

Kids have incredible compassion and are often eager to use their seemingly endless energy to help others. Youth have always participated in our fundraising activities, but now they are invited to become even more involved through some exciting new programs:

A Hero Dash is a short-distance race catering to kids under the age of 13 that can be completely customized to work best in your community or school.

Change for NF is a new program that teaches students about NF while encouraging them to turn their compassion into action by working together to collect change to help those affected by NF.

Little Heroes 5K is a short race for kids and adults. We have a great toolkit available to help you host one in your community.

All three programs require at least one adult to act as an organizer, but we are here to help, too! For more information on these programs, visit ctf.org/kids or contact Angela Earle, Community & Youth Events Manager at aearle@ctf.org or 972-587-7814.

Don't forget that your child can always participate in a **Fun Run** at one of our NF Walk events! Contact nfwalk@ctf.org to learn more.

And be on the lookout for our upcoming **Kids T-Shirt Design Contest!**



Report from NF Camp

Over 70 youth attended the 19th annual NF Camp, which took place in two sessions this July. NF Camp is held annually at the beautiful Camp Kostopolos in Emigration Canyon, Utah. Campers enjoyed horseback riding, ropes courses, whitewater rafting, and day trips to places like Boondock's Food & Fun and Lagoon Adventure Park. Campers at the second session were even treated to a surprise concert by musician and philanthropist Scott Helmer! New friendships were forged, old friendships invigorated, and brand new memories made at NF Camp 2015.

just for kids

Check out these back-to-school ice breakers:

Q: What do you call a fake noodle?

A: An impasta

Q: What do you call an alligator in a vest?

A: An investi-gator

Q: Why did the picture go to jail?

A: Because it was framed

Thanks to Brice Bakker for submitting these hilarious jokes!

FOUNDATION NEWS

NF Forum Unites NF Families

"I don't feel alone anymore!"

We frequently hear this proclamation at the conclusion of our annual NF Forum. People who live with NF can feel almost singular in their fight against the disorder. The Forum originated out of an abiding interest in the scientific discoveries discussed at the NF Conference, but it has rapidly become a place for fellowship as well.

This year's Forum proved no exception. Nearly 300 people gathered in Arizona to be inspired, to learn, and to unite as one large NF family. The Forum was preceded by CTF's first annual Foundation-wide Volunteer & Leadership Summit, designed to train and nurture active Foundation volunteers to become even stronger advocates in the fight to end NF.

The 2015 CTF Ambassador **Jeffrey Owen Hanson**, along with his parents Hal and Julie, officially kicked off the Forum with an inspirational keynote address. Their spirited and playful candor set the tone for the rest of the weekend. Children played games, learned mindfulness techniques, and made new friends, while adults attended presentations as varied as Dr. Lisa Coyne's "Acceptance Based Approach to Fostering Social Development," and Dr. Nicole Ullrich's "Understanding Clinical Trials," to breakout sessions addressing concerns specific to different manifestations of NF. Doctors Scott Plotkin, Karin Walsh, and Michael Fisher provided updates on current clinical trials, while artist and honorary national CTF board member Michie O'Day and Paralympic athlete Derrick Helton inspired with their talk, "Maximizing Physical Function in NF2."

It was a full schedule, concluding with the Scottsdale NF Walk on Sunday morning. And the weekend's impact still lingers. As attendee Nicole Steinert reflected, "What I walk away with after five days: a strong sense of family and togetherness. Sincere gratitude for those that have gone before me and paved the way in the field of NF to grow CTF to what it is today."

To see additional photos of any of the events in Scottsdale, AZ, visit ctf.org/pics



Julie, Jeffrey Owen, and Hal Hanson



The Volunteer & Leadership Summit

The Volunteer & Leadership Summit, which took place in Scottsdale, Arizona on April 15th and 16th, provided an opportunity for our active volunteers to learn how to become more effective fundraisers, grow their events, connect with other volunteers throughout the country, and learn more about the many programs the Foundation offers. Breakout sessions included information about Community Relations, Awareness Month, NF Walk, NF Endurance, Racing4Research, Cupid's Undie Run and so much more! Volunteers not only made new connections, they also became more knowledgeable about the Foundation and our efforts to end NF. For more information about how to qualify to attend next year's Volunteer & Leadership Summit, please contact your regional Community Relations representative.



NF CONFERENCE Major Announcement *Continued from page 1*

In her presentation, Dr. Widemann highlighted that the RAS/RAF/MAPK pathway is activated in NF1-related PNs, and that 16 of 24 patients treated with the MEK inhibitor selumetinib (AZD6244 hydrogen sulfate) saw a tumor volume decrease of $\geq 20\%$ and clinical improvement. This result shows that NF research has made progress not only in the identification of active agents in the preclinical model, but also in the clinic as well.

Additional preclinical and clinical work will be ongoing to direct the future development of agents for NF1 PNs, and a phase II registration trial is in development which will include evaluation of patient-reported outcome measures and functional measures, in addition to volumetric MRI analysis to evaluate if PN volume decreases can result in clinical improvement.

"It's very exciting to be able to report this significant clinical progress in the development of an effective treatment for NF1," said Dr. Widemann. "NF patients are waiting for results, and through this trial of selumetinib, and the work of Nancy Ratner and Wade Clapp, who highlighted the utility of this model through their work with MEK inhibitors, we're hopeful for the future. I'm especially grateful to the Children's Tumor Foundation, Astra Zeneca, CTEP, the NCI, and, of course, the participating patients for their support and/or funding of NF research, which is resulting in positive results for all patients with NF."

"The success reported by Dr. Widemann gives us hope," said Annette Bakker, PhD, President and Chief Scientific Officer of the Children's Tumor Foundation. "Our driving focus is on accelerating NF research through innovative collaborations, and Dr. Widemann's incredible work with plexiform neurofibromas is a great example of this model. On behalf of the NF community of patients, we give big thanks and congratulations to Dr. Widemann for her success."



Wear your support for CTF on your sleeve (or shirt, or purse, or...)

Looking for a way to show support for the NF Hero in your life? We have you covered. These 2.5" buttons are now available for sale: \$5 each OR get one of each for \$12 (sorry, no substitutions for this special pricing).

To purchase, please visit the Children's Tumor Foundation store at ctf.org/store.



ATTENTION ADULTS WITH NF

The Children's Tumor Foundation is dedicated to providing support and resources for individuals with NF of all ages. In order to do so, we want to hear from adults with NF! You are important to us and we want your thoughts and ideas on how we can support you! Please complete our short survey to let us know what is important to you and to share your suggestions for how we can continue to expand adult focused programs. The link to the survey is: ctf.org/NFAdults.



NF News is the official publication of the Children's Tumor Foundation. All issues are available on our website at www.ctf.org. Please direct any questions to info@ctf.org.

The Children's Tumor Foundation is a 501(c)(3) not-for-profit organization dedicated to finding effective treatments for the millions of people worldwide living with neurofibromatosis (NF), a term for three distinct disorders: NF1, NF2, and schwannomatosis. NF can cause tumors to grow on nerves throughout the body and may lead to blindness, deafness, bone abnormalities, learning disabilities, disabling pain, and cancer. NF affects one in every 3,000 people, more than cystic fibrosis, Duchenne muscular dystrophy, and Huntington's disease combined. The Children's Tumor Foundation funds critical research into neurofibromatosis. In addition to benefiting those who live with NF, this research is shedding new light on several forms of cancer, brain tumors, bone abnormalities, and learning disabilities, ultimately benefiting the broader community.

FOUNDATION STAFF

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Reid Horovitz, Chief Operating Officer

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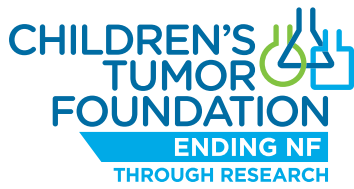
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Maggie Converse, NF Endurance Coordinator
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THE NEW YORK
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New York, NY

For more information contact
Rebecca De Ornelas
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rdeornelas@ctf.org

ctf.org/nygala



**BENEFIT 2015
GALA**

SATURDAY, OCTOBER 24
BOSTON MARRIOTT,
CAMBRIDGE, MA

**FOR MORE INFORMATION
CONTACT
KRISTINE POIRER
KPOIRER@CTF.ORG**

THE BENEFIT III

A PLAYDATE TO END NF

**Saturday,
November 7th, 2015
6:30 p.m.**
Detroit, Michigan
Cobo Center

Need more information?
Visit CTF.org/TheBenefitDetroit or
email JoyceKeller@QuickenLoans.com

